REMARKS OF
HENRY A. WAXMAN,
CHAIRMAN,
SUBCOMMITTEE ON HEALTH AND THE ENVIRONMENT
BEFORE
THE PHARMACEUTICAL INDUSTRY WASHINGTON ADVISORY COMMITTEE
MARCH 19, 1984

I'M GLAD TO BE ABLE TO JOIN YOU THIS MORNING.

AS YOU KNOW, THE CONGRESS IS IN A VERY SHORT LEGISLATIVE SESSION THIS YEAR. BUT HOWEVER SHORT THE TIME, MANY IMPORTANT ACTIONS WILL BE TAKEN.

SOME OF THEM WILL RECEIVE THE CONSIDERATION THAT THEY DESERVE. SOME OF THEM WILL NOT.

THROUGHOUT THE YEAR, HOWEVER, THERE WILL BE ENORMOUS PRESSURE TO MAKE QUICK FIXES AND TO GET SHORT-TERM SAVINGS.

- o THERE IS TALK OF COMPLETE FREEZES IN VITAL PROGRAMS.
- o THERE ARE PROPOSALS FOR MORE CUTS IN MEDICARE, CUTS THAT WILL CERTAINLY BE PASSED ON TO PATIENTS.
- o AND THERE ARE ALL THE USUAL ATTACKS ON THE MEDICAID PROGRAM.

THESE ARE NOT REALISTIC SUGGESTIONS: THE CONGRESS HAS REJECTED MOST OF THEM BEFORE AND SHOULD REJECT THEM AGAIN NOW.

I MUST ADD, ALSO, THAT DURING THIS ENTIRE YEAR, THE PRESIDENT HAS PROVIDED NO LEADERSHIP AT ALL IN HEALTH POLICY OR THE BUDGET.

HIS 1985 BUDGET WAS REJECTED OUT OF HAND, WITHOUT ANY SERIOUS CONSIDERATION BY REPUBLICANS OR DEMOCRATS. ONLY AFTER EXTENSIVE PRESSURE FROM THE REPUBLICAN SENATE DID HE EVEN BEGIN TO LOOK AT THE DEFICIT. HIS RESPONSE EVEN THEN WAS TO PASS THE PROBLEM ON FOR MEETINGS AND DISCUSSION.

HE HAS DRAGGED HIS FEET AT TAXES AND MILITARY CUTS, EVEN WHEN HIS WALL STREET ADVISORS HAVE TOLD HIM THAT SUCH MOVEMENTS ARE CRUCIAL. ONLY RECENTLY DID HE EVEN AGREE TO RE-CONSIDER WHAT HE CALLS "TAX LOOPHOLES OF QUESTIONABLE FAIRNESS."

THE RESULT IS A DEFICIT THAT THE CONGRESSIONAL BUDGET OFFICE SAYS WILL BE ALMOST $400 BILLION A YEAR BY THE END OF THE DECADE.

THIS BUDGET PRESSURE WILL BE EXPECIALLY DIFFICULT ON LEGISLATION THIS YEAR. EVERY LEGISLATIVE IDEA--FROM MEDICARE COVERAGE OF CYCLOSPORIN TO HEALTH CARE FOR THE UNEMPLOYED--WILL BE PUT TO A LITMUS TEST OF COST. EVEN HOLDING ON TO THE HEALTH PROGRAMS NOW IN PLACE WILL BE DIFFICULT.

BUT IN SPITE OF THE SHORTNESS OF THE SESSION AND THE PRESS OF THE BUDGET, A NUMBER OF LEGISLATIVE ACTIONS WILL BE TAKEN--MANY OF THEM IN AREAS OF PARTICULAR INTEREST TO YOU.

PATENT-TERM/ANDA

THE PATENT-TERM DEBATE HAS BEEN ONGOING BETWEEN US FOR SOME TIME. YOUR INDUSTRY HAS ARGUED--WITH SOME JUSTIFICATION--THAT THE REGULATORY PROCESS TAKES TIME FROM THE COMMERCIAL PATENT LIFE OF YOUR PRODUCTS, AND THAT ADDITIONAL PATENT TIME WOULD PROVIDE A SIGNIFICANT, NEW INCENTIVE TO SPEND MILLIONS TO SEARCH FOR INNOVATIVE DRUGS.

I DISAGREED WITH YOU BECAUSE I BELIEVE THAT, IN ALL BUT THE MOST EXTREME DELAYS, THE PATENT LIFE STILL OFFERS A SUFFICIENT INCENTIVE FOR CREATIVITY--AN INCENTIVE NOT VERY DIFFERENT FROM THAT OF OTHER MANUFACTURERS WHO MUST TAKE TIME TO COMMERCIALIZE THEIR INVENTIONS.

WE HAVE HAD ANOTHER DEBATE TOO--THIS ONE ABOUT GENERIC DRUGS. I HAVE BEEN CONCERNED FOR A LONG TIME THAT FDA'S POLICIES ON GENERIC DRUGS ARE INSUFFICIENT. FDA COULD CHANGE ITS POLICY TO ALLOW GENERIC VERSIONS OF POST-1962 DRUGS.

BUT IT HASN'T. IT HAS DRAGGED ITS FEET AT EVERY STEP, AT A COST TO CONSUMERS OF A BILLION DOLLARS IN THE NEXT 12 YEARS.

IT BECAME OBVIOUS TO ME THAT LEGISLATION WAS THE ONLY WAY TO SOLVE THIS PROBLEM, AND LAST SUMMER I INTRODUCED LEGISLATION TO PROVIDE FOR "ABBREVIATED NEW DRUG APPLICATIONS" FOR DRUGS APPROVED AFTER 1962. YOUR INDUSTRY TOOK ISSUE WITH MY BILL--ARGUING AGAIN THAT THERE ARE INADEQUATE INCENTIVES TO FIND NEW DRUGS. EASIER APPROVALS OF GENERICS, YOU SAID, WOULD UNDERMINE YOUR ABILITY TO INCREASE YOUR INVESTMENT IN R AND D.

AT THAT POINT DISCUSSIONS WITH MANY OF YOU BEGAN, AND YOU PROVIDED ME WITH THE ESSENTIAL PATENT DATA FOR DRUGS APPROVED SINCE 1962. AFTER EVALUATING THAT DATA I WAS PREPARED TO MOVE FORWARD AND I AM HAPPY TO SAY THAT MEETINGS WITH LEW ENGMAN, REPRESENTING THE PMA, HAVE BEEN SUCCESSFUL AND THAT A COMBINATION OF GOALS HAS BEEN MADE. WE HAVE COMBINED THE FORCE BEHIND PATENT-TERM EXTENSION LEGISLATION AND GENERIC DRUG PROPOSALS, AND I THINK WE HAVE A PACKAGE THAT IS BALANCED AND IN EVERYONE'S INTEREST.

EVERYONE, BUT PARTICULARLY THE ELDERLY WHO USE 25 PERCENT OF ALL PRESCRIPTION DRUGS, WILL BENEFIT TWICE. FIRST, THEY GET LOWER DRUG PRICES BECAUSE GENERICS ARE AVAILABLE, AND THEN THEY GET IMPORTANT NEW DRUGS THEY SO DESPERATELY NEED. IN ADDITION, THE LEGISLATION WILL HELP ASSURE THAT THE U.S. PHARMACEUTICAL INDUSTRY MAINTAINS THE LEAD IN THE WORLD MARKETPLACE.

I CONGRATULATE THE PHARMACEUTICAL INDUSTRY--BOTH BRAND AND GENERIC--FOR YOUR SUPPORT FOR THIS LEGISLATION.

I KNOW YOU ARE ALL INTERESTED IN OUR PROGRESS. DEVELOPMENT OF THE LEGISLATIVE LANGUAGE OF THE BILL IS WELL UNDERWAY. I LOOK FORWARD TO YOUR HELP IN PASSAGE, AND I MUST ADD I LOOK FORWARD TO THE RESEARCH AND DEVELOPMENT THAT WILL--I TRUST--RESULT FROM THIS EXTENSION OF MARKETING OF BRAND-NAME DRUGS.

ORPHAN DRUGS

LET ME ALSO GIVE YOU A BRIEF REPORT ON THE PROGRESS OF THE ORPHAN DRUG ACT OF LAST YEAR. I BELIEVE THAT PROGRAM IS A PRIME EXAMPLE OF THE COOPERATION BETWEEN THE CONGRESS AND THE DRUG INDUSTRY TOWARD COMMON GOALS. I'M PROUD OF IT, AND I THINK YOU CAN BE, TOO.

THE SUBCOMMITTEE WILL BE HOLDING ITS FIRST OVERSIGHT HEARING ON THAT LAW THIS FRIDAY. THE HEARING WILL EVALUATE THE SUCCESS OF THE ACT IN ENCOURAGING THE DEVELOPMENT OF NEW DRUGS FOR RARE DISEASES.

WE WILL BE HEARING FROM MANUFACTURERS AND FROM THE DISEASE GROUPS ABOUT PROBLEMS WITH THE BILL. YOU KNOW, I'M SURE, THAT THE REGULATIONS FOR THE TAX CREDIT PROVISIONS ARE STILL BEING HELD AT THE TREASURY DEPARTMENT. YOU MAY ALSO KNOW THAT THE SMALL GRANTS SECTION FOR INDEPENDENT RESEARCHERS DOESN'T PROVIDE FUNDING FOR THE COSTS OF ANIMAL RESEARCH.

I AM VERY CONCERNED ABOUT BOTH THESE PROBLEMS, AND WE WILL BE LOOKING FOR SOLUTIONS.

THE HEARINGS WILL ALSO RECOGNIZE THE ACTIONS TAKEN BY PRIVATE GROUPS:

THE PMA'S COMMISSION ON DRUGS FOR RARE DISEASES;

THE GENERIC PHARMACEUTICAL INDUSTRY'S ORPHAN DRUG INSTITUTE; AND

THE NATIONAL ORPHAN DRUG AND DEVICES FOUNDATION CHAIRED JOEL BENNETT.

I CAN TELL YOU NOW THAT WE ARE PLEASED WITH THE PROGRESS SO FAR. TWENTY-FOUR ORPHAN DRUGS, NOT ON THE MARKET, ARE BEING DEVELOPED. THREE OTHER MARKETED DRUGS ARE BEING DEVELOPED FOR ORPHAN USES.

IN ONE YEAR, THAT'S A REAL ACHIEVEMENT. ONCE THE TAX CREDIT IS IN PLACE, I HAVE HIGH HOPES THAT EVEN MORE CAN BE DONE.

CYCLOSPORIN

A THIRD AREA THAT I KNOW IS OF SPECIAL INTEREST TO YOU IS THE PROGRESS OF LEGISLATION REGARDING ONE PARTICULAR DRUG--CYCLOSPORIN.

AS I'M SURE YOU KNOW, CYCLOSPORIN HAS REVOLUTIONALIZED ORGAN TRANSPLANTATION. IT HAS DRAMATICALLY IMPROVED GRAFT SURVIVAL RATES FOR KIDNEY TRANSPLANTS AND HAS ELEVATED HEART AND LIVER TRANSPLANTS BEYOND CLINICAL EXPERIMENTATION. THIS DRUG HAS REDUCED THE INCIDENCE OF ORGAN REJECTION AND ENABLED SUBSTANTIAL INCREASES IN THE NUMBER AND SUCCESS OF TRANSPLANT OPERATIONS.

WHEN THE COMMERCE COMMITTEE REPORTED THE "NATIONAL ORGAN TRANSPLANT ACT," A PROVISION WAS ADDED TO ALLOW MEDICARE TO REIMBURSE FOR THE COSTS OF IMMUNOSUPRESSANT DRUGS PATIENTS DEPEND UPON AFTER THEIR TRANSPLANT SURGERY. THESE DRUGS ARE ESSENTIAL IF AN ORGAN TRANSPLANT IS TO BE SUCCESSFUL.

ALTHOUGH MEDICARE WILL PAY FOR THE COSTS OF A KIDNEY TRANSPLANT IT WILL NOT COVER THE COSTS OF MEDICATIONS WHICH WILL PREVENT THE ORGAN FROM BEING REJECTED FOLLOWING SURGERY. SINCE THESE DRUGS -- PARTICULARLY CYCLOSPORIN -- ARE EXPENSIVE AND MUST BE TAKEN FOR AN INDEFINITE PERIOD OF TIME, THEIR COSTS CAN BE A BURDEN FOR PATIENTS. IN MANY CASES THE BURDEN IS SO GREAT AS TO REQUIRE A PATIENT TO TAKE A LESS EFFECTIVE MEDICATION AND RISK MEDICAL COMPLICATIONS.

BY NOT PAYING FOR THESE DRUGS, MEDICARE IS IMPEDING THE MOST COST-EFFECTIVE AND CLINICALLY EFFECTIVE FORM OF TREATMENT. WE KNOW THAT KIDNEY DIALYSIS IS MORE EXPENSIVE THAN TRANSPLANTATION.

WHEN THE COMMERCE COMMITTEE MET TO CONSIDER THIS ISSUE THE CONGRESSIONAL BUDGET OFFICE TOLD US THAT THERE WERE SOME COSTS FOR THE PROVISION, BUT OVERALL, THE PROVISION WOULD SAVE MONEY.

AS OFTEN HAPPENS, HOWEVER, THE CBO HAS RE-CALCULATED ITS ESTIMATES AND TOLD THE WAYS AND MEANS COMMITTEE THAT THERE MAY BE SIGNIFICANT NEW COSTS. I BELIEVE IT WAS FOR THIS REASON THAT THE WAYS AND MEANS SUBCOMMITTEE ON HEALTH VOTED TO STRIKE THE CYCLOSPORIN AMENDMENT FROM THE BILL.

THERE ARE CONCERNS OVER HOW THE COSTS AND SAVINGS ATTRIBUTED TO CYCLOSPORIN ARE CALCULATED. BUT I CONTINUE TO BELIEVE THAT THIS PROVISION WOULD CONSTITUTE GOOD MEDICINE AND SOUND ECONOMICS.

THE BILL MUST STILL GO THROUGH THE FULL WAYS AND MEANS COMMITTEE AND THE HOUSE, BUT WITH THE PRESSURE TO CUT MEDICARE, ADDING NEW COSTS WILL BE VERY DIFFICULT. PROVIDING COVERAGE FOR CYCLOSPORIN WILL BE AN UPHILL FIGHT.

LABELING

FINALLY LET ME DESCRIBE THE FDA APPROVAL LABELING ACT, A BILL I INTRODUCED EARLIER IN THE YEAR. PRESENT LAW PROHIBITS ANYONE FROM MAKING REPRESENTATIONS REGARDING FDA APPROVAL IN THE LABELING OR ADVERTISING OF ANY DRUG OR DEVICE.

THIS STATUTORY BAN MAKES IT DIFFICULT FOR PHARMACISTS TO DETERMINE WHETHER A DRUG HAS BEEN APPROVED BY FDA. ALTHOUGH IT IS ILLEGAL TO MARKET A DRUG WITHOUT PRIOR FDA APPROVAL, AND FDA AGGRESSIVELY ENFORCES THE LAW, SOME UNAPPROVED DRUGS ARE AVAILABLE IN THE MARKETPLACE AND HAVE BEEN INADVERTENTLY DISPERSED BY PHARMACISTS. IN AT LEAST ONE CASE, A PHARMACIST HAS BEEN HELD LIABLE FOR THE ADVERSE EFFECTS OF THE DRUG.

PRESENTLY, THERE IS NO SIMPLE WAY FOR A PHARMACIST FILLING A PRESCRIPTION TO CHECK WHETHER A DRUG HAS BEEN APPROVED BY FDA. WHILE MANY DRUGS ARE LABELED WITH THE NATIONAL DRUG CODE NUMBER, THAT NUMBER IS CHOSEN BY THE MANUFACTURER TO IDENTIFY THE PRODUCT AND FIRM. IT DOES NOT INDICATE FDA APPROVAL. THE NEW DRUG APPLICATION OR AN ABBREVIATED NEW DRUG APPLICATION NUMBER ALSO DOES NOT INDICATE APPROVAL, BUT JUST THAT FDA HAS RECEIVED AN APPLICATION.

THE BILL I'VE INTRODUCED WOULD ELIMINATE THE STATUTORY BAN WITH RESPECT TO DRUGS AND WOULD PERMIT ACCURATE STATEMENTS CONCERNING FDA APPROVAL IN LABELING OR ADVERTISING FOR DRUGS. FALSE OR MISLEADING STATEMENTS WOULD CONTINUE TO BE PROHIBITED.

THE BILL HAS PASSED THE HOUSE AND IS AWAITING CONSIDERATION IN THE SENATE.

CONCLUSION

AS YOU CAN SEE, THERE WILL BE A GREAT DEAL OF ACTIVITY IN PHARMACEUTICAL LEGISLATION. I CAN ASSURE YOU THAT OTHER AREAS OF PUBLIC HEALTH AND FINANCE ARE AT LEAST AS BUSY.

I'M PLEASED TO HAVE HAD THE OPPORTUNITY TO TALK TO YOU, AND I HOPE THAT WE WILL CONTINUE TO WORK TOGETHER THIS YEAR AND MAKE THE SHORT YEAR WORTHWHILE.

THANK YOU.